

Research Update from the Faustman Lab

We are very pleased to report exciting news from the Faustman Laboratory. They are progressing on schedule toward clinical trials with a drug that may help remove the disease causing cells associated with type 1 diabetes. These targeted cells are abnormal, autoreactive T cells (a form of white blood cell), which attack and destroy the special cells in the pancreas responsible for producing insulin. This is a trial for people who already have Type 1 diabetes and the goal is to reverse the disease by using a generic drug, BCG, which is already approved in the US for other disease indications and thus has a safe toxicity profile. The goal of using BCG is to target a pathway that kills these abnormal T cells hopefully allowing regeneration of the insulin producing function within the pancreas and then restoring normal regulation of blood sugar levels.

As part of the effort to translate diabetic mouse cures to human cures for diabetes, the Faustman Laboratory is devising blood tests to establish an effective dose of BCG in humans. These blood tests require two development steps. First, the Faustman laboratory must separate T cells from whole blood. For the past year, this important step has been successfully standardized using a proprietary process developed in the Faustman laboratory that removes T cells from blood with high yield, viability and purity. Unlike traditional methods, this process is highly amenable towards automation. This process has been validated in over 266 human blood samples (half of those from type 1 diabetics), exceeding the goal of 50 human samples by this year. Secondly, the isolated T cells will then be used in developing clinical assays to quantify the numbers of autoreactive cells before and after treatment with BCG

The Faustman Laboratory has also been successful in automating this T cell separation process achieving higher degrees of standardization and consistency suitable for conducting the upcoming clinical trials, which will commence in 2008.

The Iacocca Foundation has made payment for year two funding of this exciting project at the Massachusetts General Hospital. In year two, the funding for the Nathan/Faustman research program will be aimed to translate the diabetes 'cure' from mice to humans. The goals will be to: 1) recruit more type I diabetes patients; 2) implement a clinical blood test to characterize the T cell defect in recruited patients; 3) continue drug dosing studies in the NOD diabetic mouse model; and 4) start the BCG administration of drug to patients.

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